THE NEED FOR GREATER REFLECTION OF HETEROGENEITY IN CEA

or
(The ***fLaw*** of averages)

Warren Stevens PhD

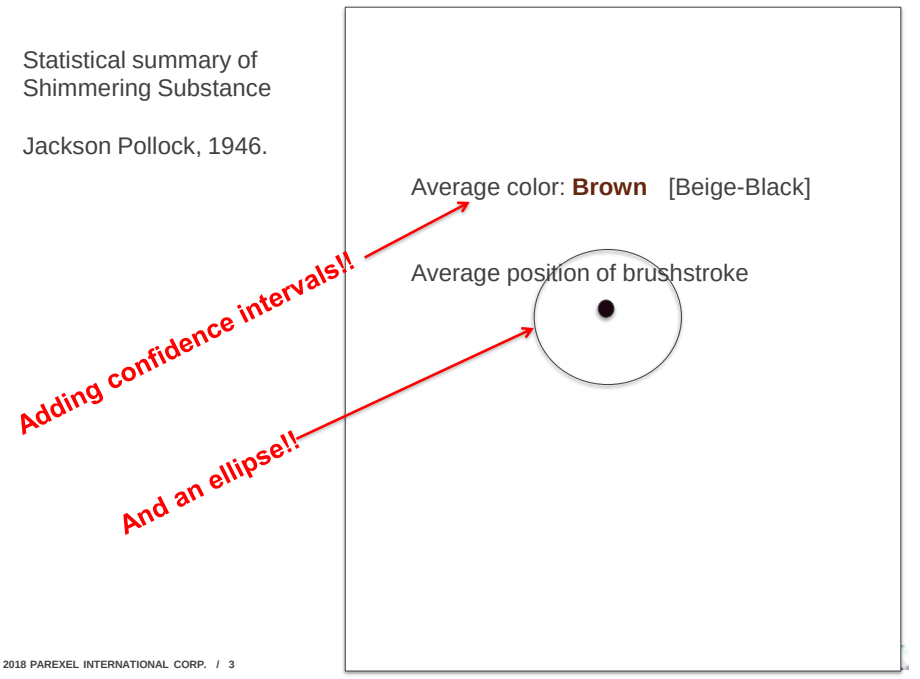
PAREXEL
YOUR JOURNEY. OUR MISSION.

Shimmering Substance

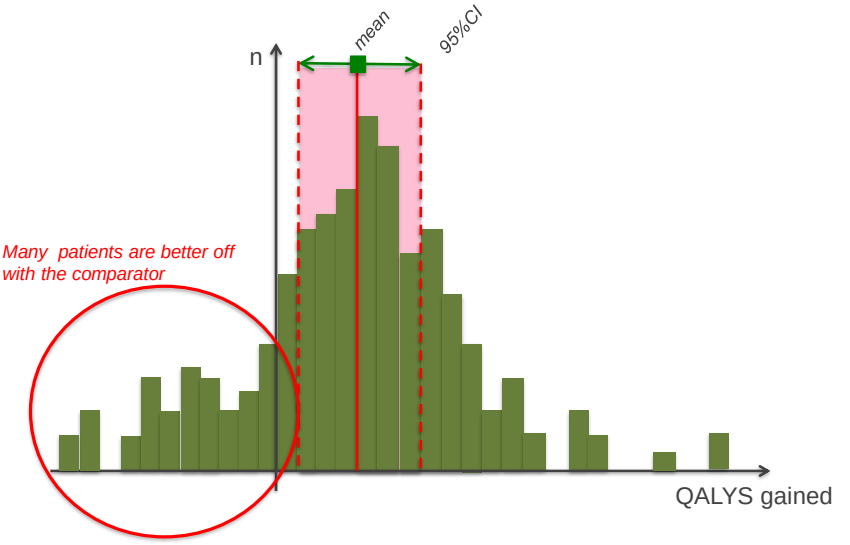
Jackson Pollock, 1946.

Statistical summary of
Shimmering Substance

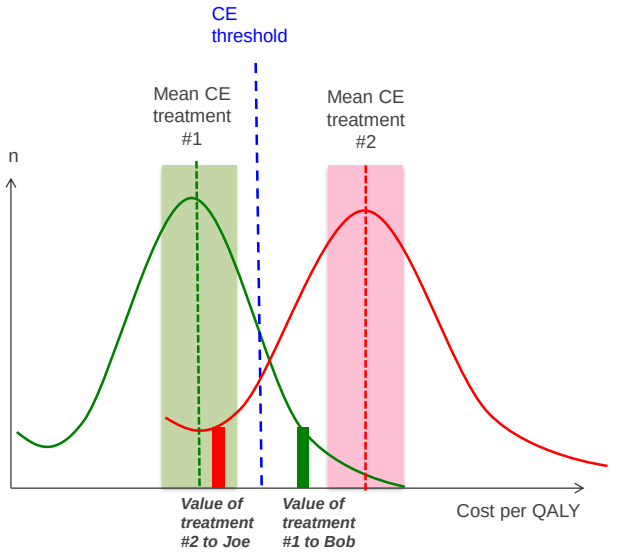
Jackson Pollock, 1946.



WE KNOW DATA ON EFFECTIVENESS IS FAR MORE COMPLEX THAN WE LIKE TO REPRESENT IT



A THOUGHT EXPERIMENT



A THOUGHT EXPERIMENT

- Joe is receiving a treatment that is cost-effective for him, but deemed un cost-effective by/for society
- Bob is receiving an treatment that is not cost-effective for him, but has been deemed cost-effective by/for society

- So who's treatment is cost-effective?

HYPOTHETICALLY THERE ARE AS MANY LEVELS OF EFFECTIVENESS (OR COST-EFFECTIVENESS) AS THERE ARE PATIENTS

What questions or uncertainties stop us recognizing this basic truth in scientific practice?

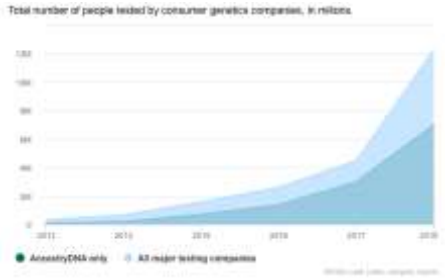
1. **Uncertainty (empiricism) –** how do we gauge uncertainty in a sample size of 1? avoiding false positives
2. **Policy –** how do we institute a separate treatment policy for each and every individual?
3. **Information (data) –** how we do make 'informed' decisions without the level of detail required in terms of information (we know more about *populations* than we do about *people*)

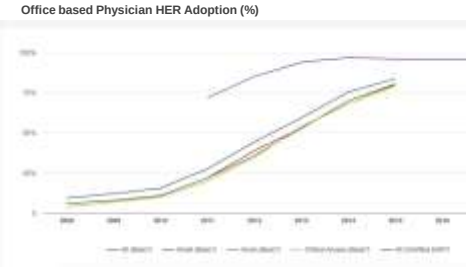
BUT THERE ARE ALSO A NUMBER OF VERY REAL QUESTIONS WE MUST CONSIDER AS ECONOMISTS;

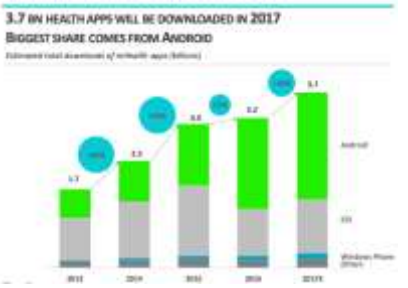
1. ***Relevance*** – why institute a policy, or deliver a therapy, that we know wont be effective (or cost-effective) for a good proportion of the patients who receive it?
2. ***Policy logic*** – why have a system that aims for resource optimization, but accepts that much of its resources are ***systematically*** wasted
3. ***Data application*** - Why collect tons of evidence of the varying levels of effectiveness of an intervention across a heterogeneous population and use only a fraction of it?

IF THE QUESTION OF OF REFLECTING HETEROGENEITY IN EVIDENCE HAS BEEN AROUND FOREVER WHY ADDRESS IT NOW?

DEMAND: THE AGE OF BIG DATA



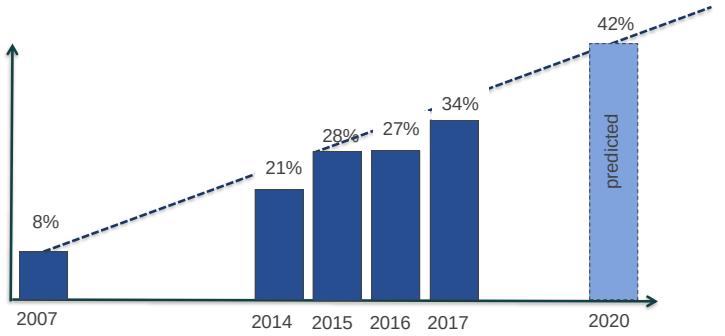




Source: The Office of the National Coordinator for Health IT

SUPPLY: CHANGES IN TARGETED NATURE OF NEW THERAPIES

Proportion of new drugs that are PM approved by FDA by year



Source: Personalized Medicine at FDA: Progress Report 2017

THE WINNERS AND THE WINNERS: WHO GAINS AND WHO GAINS FROM GREATER REFLECTION OF HOE?

	Manufacturers	Payers	Patients / Society
Short term / narrow scope (1)	• **Improved mean ICERs** • Higher chance of approval / reimbursement	• Better selection of therapeutic options • Less waste • More efficient use of resources	• More therapeutic options available • Healthcare geared more towards the individual
Medium term / scope (2)	• Higher average prices • Perhaps a smaller market but also this may be countered by higher scope of indications (TBC)	• Greater availability of treatment options within therapeutic areas • **Greater efficiency of available treatment options within therapeutic areas**	• More choice • Improved efficiency of health care resources
Long term / wide scope (3)	• **More drugs approved** • **Higher average prices** • Higher scope of indications • Greater spending on newer drugs (as a share of all drugs) – due to evidence of greater net gains from new drugs • Greater spending on all drugs (as a share of healthcare spend)	• More efficient uses of resources • More cost-effective healthcare spending • **Less waste (lower use of drugs where they don't add value)** • Greater set of effective tools for patient benefit • Value-based pricing with Pharma (less risk)	• **Greater overall health gains within limited resource system** • **More efficient use of health care resources** • **More equitable distribution of health gains**

1. Brand / drug level value in terms of added value to manufacturer / sponsor either through gains in scope of indications, price or utilization over competitors
2. Overarching therapeutic area value relevant to 'drug group' in specific system or globally
3. Wider benefits that are not specific to a particular therapeutic area, but a whole disease or a whole health system

WHERE TO NEXT?

Once we start to accept the essential need for reflecting heterogeneity in cost-effectiveness it's a short hop to heterogeneity in 'valuing' health

Two people may value particular health states very differently?

- Length of life versus quality of life?

- Physical functioning over mental acuity?

IN SUMMARY

- The development of CE methods owes as much to the desire to be accepted in medicine and medical publications as to the goals of the science itself: optimization of scarce resources
  - As a result we tend to lean too heavily on population statistics and the imagined homogenous blob of being a 'people'
- True heterogeneity exists and not reflecting it ultimately risks greater than necessary inefficiency and inequality in healthcare provision
- If we are systematic about this approach everybody gains; patients, manufacturers and payers
- One of the biggest barriers to methods reflecting heterogeneity of effect in CE and policy has been a lack of real, consistently high quality, patient specific data - that time is coming to an end